

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property Organization
International Bureau(43) International Publication Date
12 April 2001 (12.04.2001)

PCT

(10) International Publication Number
WO 01/25452 A1(51) International Patent Classification⁷: C12N 15/64,
15/85, 5/10, A61K 31/70, 48/00

(21) International Application Number: PCT/GB00/03806

(22) International Filing Date: 4 October 2000 (04.10.2000)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
9923381.9 4 October 1999 (04.10.1999) GB

(71) Applicant and

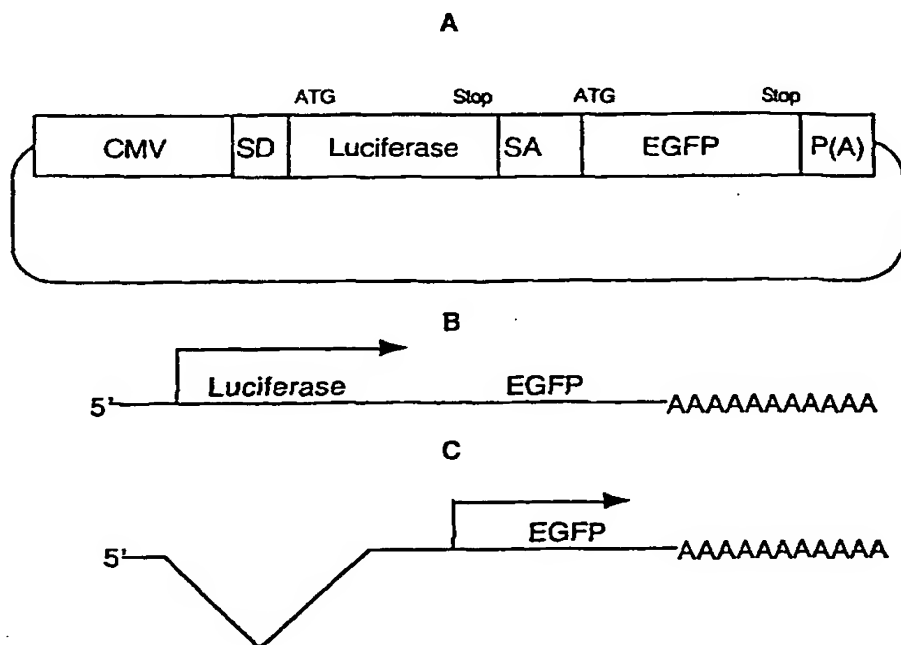
(72) Inventor: BROSENS, Jan [BE/GB]; 29 Carthew Road,
London W6 0DU (GB).(74) Agents: GARDENER, Rebecca et al.; Frank B. Dehn &
Co., 179 Queen Victoria Street, London EC4V 4EL (GB).(81) Designated States (*national*): AE, AG, AL, AM, AT, AT
(*utility model*), AU, AZ, BA, BB, BG, BR, BY, BZ, CA,CH, CN, CR, CU, CZ, CZ (*utility model*), DE, DE (*utility model*), DK, DK (*utility model*), DM, DZ, EE, EE (*utility model*), ES, FI, FI (*utility model*), GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KR (*utility model*), KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NO, NZ, PL, PT, RO, RU, SD, SE, SG, SI, SK, SK (*utility model*), SL, TJ, TM, TR, TT, TZ, UA, UG, US, UZ, VN, YU, ZA, ZW.(84) Designated States (*regional*): ARIPO patent (GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GW, ML, MR, NE, SN, TD, TG).

Published:

— With international search report.

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: TARGETED GENE THERAPY



(57) Abstract: The present invention relates to a genetic construct which comprises an NOI and splice-functional element(s) selected and arranged such that the first ORF available for translation in a target cell population is for the NOI while in non-target cells the NOI ORF is not the primary ORF, such constructs for use in therapy and the use of splice-functional elements to restrict translation or expression of a nucleotide of interest to specific cell types and/or environments.

WO 01/25452 A1